CytRx Highlights Orphazyme's Published Results from its Phase 2/3 Trial of Arimoclomol in Niemann-Pick Disease Type C

12-Month Study of Arimoclomol Showed a Clinically Meaningful Treatment Effect, Corresponding to a Reduction in Disease Progression

Highlights Arimoclomol is Under Regulatory Review in Europe, With a Decision Expected in Q4 2021

Notes Orphazyme Stated it Continues to Evaluate a Path Forward for Arimoclomol in the U.S.

LOS ANGELES--(BUSINESS WIRE)--CytRx Corporation (OTCQB:CYTR) ("CytRx" or the "Company"), a specialized biopharmaceutical company focused on research and development for the oncology and neurodegenerative disease categories, today highlighted that Orphazyme A/S (NASDAQ: ORPH) ("Orphazyme") published the results from a Phase 2/3 trial of arimoclomol, an investigational heat-shock protein amplifier, in Niemann-Pick disease type C (NPC) in the peer-reviewed Journal of Inherited Metabolic Disease (JIMD). The online publication is available here.

The Phase 2/3 trial (NPC-002; ClinicalTrials.gov identifier: NCT02612129), was a prospective, randomized, double-blind, placebo-controlled study. Fifty patients aged 2–18 years were randomized 2:1 to arimoclomol:placebo, stratified by miglustat use. Routine clinical care was maintained. Arimoclomol was administered orally three times daily. The primary endpoint was change in 5-domain NPC Clinical Severity Scale (NPCCSS) score from baseline to 12 months, as described by Mengel et al.[1] and Patterson et al[2]. The 5-domain NPCCSS comprises the domains determined to be most clinically relevant to patients, caregivers, and clinicians: ambulation, cognition, fine motor skills, speech, and swallowing (Cortina-Borja et al.[3]). A recent validation of the 5-domain NPCCSS shows that a change of 1 point or greater on the total score constitutes a clinically meaningful change for caregivers/patients and physicians (Patterson et al[4]).

At 12-months, a significant treatment effect in favor of arimoclomol of −1.40 points (95% CI: −2.76, −0.03; p = 0.046) was observed, corresponding to a 65% relative reduction in annual disease progression. In the prespecified subgroup of patients receiving miglustat as routine care, arimoclomol resulted in stabilization of disease severity with a treatment difference of −2.06 in favor of arimoclomol (p = 0.006). In the pre-specified subgroup of patients ≥4 years of age the mean treatment difference was −1.80 in favor of arimoclomol (p=0.016), corresponding to 82% relative reduction in annual disease progression.

Arimoclomol was well-tolerated, with adverse events occurring in 88.2% of patients receiving arimoclomol and 75.0% of patients receiving placebo. Fewer patients had serious adverse events with arimoclomol (14.7%) versus placebo (31.3%).

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[1] Mengel E, Bembi B, Del Toro M, et al (2020) Clinical disease progression and biomarkers in Niemann–Pick disease type C: a prospective cohort study. Orphanet J Rare Dis 15: 328.
[2] Patterson MC, Lloyd-Price L, Guldberg C, et al (2021) Validation of the 5-domain Niemann-Pick type C Clinical Severity Scale. Orphanet J Rare Dis 16: 79.
[3] Cortina-Borja M, Vruchte D, Mengel E, et al (2018) Annual severity increment score as a tool for stratifying patients with Niemann-Pick disease type C and for recruitment to clinical trials. Orphanet J Rare Dis 13: 143. doi:110.1186/s13023-13018-10880-13029.
[4] Patterson MC, Lloyd-Price L, Guldberg C, et al (2021) Validation of the 5-domain Niemann-Pick type C Clinical Severity Scale. Orphanet J Rare Dis 16: 79.

Orphazyme's Chief Medical Officer stated the following in an announcement this week:

"We are pleased to share the data from our Phase 2/3 trial in JIMD. NPC is a rare, inherited progressive neurodegenerative disorder with a high unmet medical need for disease-modifying treatment options. This trial demonstrated a statistically significant and clinically meaningful treatment effect of arimoclomol in NPC supported by significant and consistent effects across several disease- and pharmacodynamic biomarkers. We believe these data establish the potential of arimoclomol as an efficacious and well-tolerated disease-modifying treatment for NPC."

Orphazyme's Chief Executive Officer added the following in an announcement this week:

"We are committed to serving the NPC community and are working expeditiously to deliver this potential new medicine to patients. Arimoclomol is under regulatory review in Europe, with an anticipated CHMP opinion in Q4 2021, and we continue to evaluate the path forward in the U.S. following the recent FDA response."

Steven A. Kriegsman, Chairman and Chief Executive Officer of CytRx, commented:

"CytRx is encouraged by Orphazyme's published results of its Phase 2/3 trial of arimoclomol in Niemann-Pick disease type C, which exhibited a statistically significant and clinically meaningful treatment effect on reducing disease progression. The biomarker data further suggests arimoclomol can be an effective solution for young patients suffering from NPC, with relatively few serious adverse effects. We look forward to monitoring Orphazyme's pursuit of European regulatory approval for arimoclomol in Q4 2021 and additional developments in the U.S. following the FDA's feedback."

About CytRx

CytRx Corporation (OTCQB: CYTR) is a biopharmaceutical company with expertise in discovering and developing new therapeutics principally to treat patients with cancer and neurodegenerative diseases. CytRx's most recent advanced drug conjugate, aldoxorubicin, is an improved version of the widely used anti-cancer drug doxorubicin and has been out-licensed to ImmunityBio, Inc. (NASDAQ: IBRX). In addition, CytRx's drug candidate, arimoclomol, was sold to Orphazyme A/S (Nasdaq: ORPH) in exchange for milestone payments and royalties. Orphazyme is developing arimoclomol in Niemann-Pick disease Type C ("NPC") and Gaucher disease. Learn more at www.cytrx.com.

Forward-Looking Statements

This press release contains forward-looking statements. These statements are not historical facts, but instead represent only CytRx's belief regarding future events, many of which, by their nature, are inherently uncertain and outside of CytRx's control. Forward-looking statements include statements relating to the potential receipt of EMA and FDA approval of arimoclomol and the CytRx's potential receipt of future milestone and royalty payments from Orphazyme. Such statements involve risks and uncertainties that could cause actual events or results to differ materially from the events or results described in the forward-looking statements, including risks and uncertainties relating to the ability of Orphazyme to obtain regulatory approval for, manufacture and commercialize its products and therapies that use arimoclomol; the results of clinical trials involving arimoclomol; the amount, if any, of future milestone and royalty payments that we may receive from Orphazyme; and other risks and uncertainties described in the most recent annual and quarterly reports filed by the CytRx with the SEC, including disclosures under the heading "Risk Factors", and current reports filed since the date of the CytRx's most recent annual report. All forward-looking statements are based upon information available to the CytRx on the date the statements are first published. The CytRx undertakes no obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

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